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PTC Therapeutics CARDINALS trial

A randomised double-blind, placebo-controlled parallel study to assess the efficacy, safety, tolerance, pharmacokinetics and biomarker effects of PTC-857 in adult patients with Amyotrophic Lateral Sclerosis.

This phase II study is sponsored by PTC Therapeutics Inc and aims to assess whether PTC-857 (or utreloxastat®) can inhibit disease progression in ALS.

In an ALS mouse model study, PTC857 was shown to protect the innervation of the neuromuscular junction in the lumbar spinal cord.

Patients with ALS who are between 18 and 80 years old, have been ill for less than 24 months, are being treated with a stable dose of riluzole (100mg/day), have a lung function (SVC) of ≥ 60% and have a score of 34 or more on the ALS-FRS-R scale at screening visit may be eligible to participate in this study. A total of 258 patients will be recruited internationally with a randomisation ratio of 2:1 PTC-857 versus placebo.

The study medication is an oral solution and should be taken twice a day during meals.

The study consists of 4 periods; a screening period of 8 weeks, a double-blind treatment period of 24 weeks, a long-term open-label treatment period of 28 weeks and finally a telephone contact 4 weeks after the last dose in the follow-up period.

More info on this study can be found on the **clinicaltrials.gov website.**

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